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Genotyping of *Smn* knockout and *SMN*^{C} transgenic mice

Smn knockout mice were genotyped by Southern blot or PCR analysis. Probes A and B, as shown in FIG. 1, were used in Southern blot analysis of mouse genomic DNA diagested with *Nhe*I or *Eco*RV. Three primers were prepared for the PCR analysis: S1 (5'-ATAACACCACCACTCTTACTC-3') (SEQ ID NO 1), S2 (5'-GTAGCCGTGATGCCATTGTCA-3'; 1,150 bp) (SEQ ID NO 2), H1 (5'-AGCCTGAAGAACGAGATCAGC-3'; 950 bp) (SEQ ID NO 3). S1 and S2 were used for detection of the wild type alleles while S1 and H1 were used for detection of the knockout alleles. Further, another three sets of PCR primers were prepared for identifying transgenic mice: (1) 5'-ACTGCAACCTCCTGGGTTCAAGTG-3' (SEQ ID NO 4) and 5'-CAGTTCGAGACCAGCCTGACCAAT-3' (SEQ ID NO 5), probing the 5' untranslated region of *SMN*; (2) 5'-CGAATCACTTGAGG GCAGGAGTTTG-3' (SEQ ID NO 6) and 5'-AACTGGTGGACATGGCTGTTCATTG-3' (SEQ ID NO 7), probing the 3' end of BAC clone; and (3) 5'-AAACCAGTCGGGCACAATACCTAGC-3' (SEQ ID NO 8) and 5'-TATGCTGATTGAAGGGAGGGGTGC-3' (SEQ ID NO 9), probing the 5' end of the BAC clone. The number of transgene copies in the transgenic mouse genome was determined in a Southern blot analysis by the amount of the probe (spanning *SMN* cDNA exons 3 and 4) that was hybridized to *Pst*I-digested mouse genomic DNA.

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Analysis of *SMN*^{C} transgene expression in mice.

Reverse transcription from total RNA was carried out using a random primer 5'-TN_{10}-3' and MMLV reverse transcriptase (from Promega). The resulting single-stranded cDNA was then PCR-amplified using one or three pairs of primers that covers the entire *SMN* coding region. The first primer pair, amplifying the fragment from the 5' untranslated region to exon 4, was a forward primer 5'-CGCTGCGCATCCGCGGGTTTGCTATGGC-3' (SEQ ID NO 10) (also referred as P1) and a reverse primer 5'-TCCCAGTCTTGGC-CCTGGCAT-3'(SEQ ID NO 11). The second primer pair, amplifying exons 4-6, was a forward primer 5'-AACATCAAGCCCAAATCTGC-3'(SEQ ID NO 12) and a reverse primer 5'-GCCAGTATGATAGCCACTCATGTACCATG-3'(SEQ ID NO 13). The third primer pair, amplifying exons 6-8 was a forward primer 5'-CTCCCATATGTCCAGATTCTCTTGATGATGC-3'(SEQ ID NO 14) and a reverse primer 5'-ACTGCCTCACCACCGTGCTGG-3'(SEQ ID NO 15) (also referred as P6). In addition, P1 and P6 were used to amplify the full-length *SMN* cDNA.